

## 2018 Current Fiscal Year Report: Muscular Dystrophy Coordinating Committee

Report Run Date: 06/05/2019 10:31:38 AM

### 1. Department or Agency

Department of Health and Human Services

### 2. Fiscal Year

2018

### 3. Committee or Subcommittee

Muscular Dystrophy Coordinating Committee

### 3b. GSA Committee No.

13889

### 4. Is this New During Fiscal Year?

No

### 5. Current Charter

08/15/2018

### 6. Expected Renewal Date

08/15/2020

### 7. Expected Term Date

### 8a. Was Terminated During Fiscal Year?

No

### 8b. Specific Termination Authority

### 8c. Actual Term Date

### 9. Agency Recommendation for Next Fiscal Year

Continue

### 10a. Legislation Req to Terminate?

Not Applicable

### 10b. Legislation Pending?

Not Applicable

### 11. Establishment Authority

Statutory (Congress Created)

### 12. Specific Establishment Authority

42 USC 283g, section 404E

### 13. Effective Date

12/18/2001

### 14. Committee Type

Continuing

### 14c. Presidential?

No

### 15. Description of Committee

Scientific Technical Program Advisory Board

### 16a. Total Number of Reports

No Reports for this Fiscal Year

### 17a. Open Meetings and Dates 3 17b. Closed Meetings and Dates 0 17c. Partially Closed Meetings and Dates 0 17d. Total Meetings and Dates 3

#### Purpose

Program Advisory

#### Start

12/05/2017

#### End

- 12/05/2017

Program Advisory

04/30/2018

- 04/30/2018

Program Advisory

09/17/2018

- 09/17/2018

### Number of Committee Meetings Listed: 3

	Current FY	Next FY
18a(1). Personnel Pmts to Non-Federal Members	\$1,600.00	\$2,400.00
18a(2). Personnel Pmts to Federal Members	\$0.00	\$0.00
18a(3). Personnel Pmts to Federal Staff	\$62,548.00	\$63,736.00
18a(4). Personnel Pmts to Non-Member Consultants	\$600.00	\$400.00
18b(1). Travel and Per Diem to Non-Federal Members	\$1,700.00	\$3,700.00
18b(2). Travel and Per Diem to Federal Members	\$0.00	\$0.00
18b(3). Travel and Per Diem to Federal Staff	\$0.00	\$0.00
18b(4). Travel and Per Diem to Non-member Consultants	\$2,619.00	\$2,800.00
18c. Other(rents,user charges, graphics, printing, mail, etc.)	\$8,520.00	\$10,300.00

<b>18d. Total</b>	\$77,587.00	\$83,336.00
<b>19. Federal Staff Support Years (FTE)</b>	0.30	0.30

**20a. How does the Committee accomplish its purpose?**

The Muscular Dystrophy Coordinating Committee (MDCC) coordinates research activities across the National Institutes of Health, with other Federal health programs and voluntary health organizations, and activities relating to the various forms of muscular dystrophy, including Duchenne, myotonic, fasioscapulohumeral muscular dystrophy. The Committee has developed a plan for conducting and supporting research and education on muscular dystrophy through the national research institutes and other member organizations of the MDCC. The Committee will determine strategies for implementation of the plan, and will periodically review and revise the plan.

**20b. How does the Committee balance its membership?**

The Muscular Dystrophy Coordinating Committee consists of 18 members, including the Chair. Two-thirds of the members represent governmental agencies, including the directors or their designees of four of the national research institutes that support muscular dystrophy research, representatives of other Federal departments and agencies whose programs support research on the muscular dystrophies or provide services to people with these diseases including the Centers for Disease Control and Prevention, the Health Resources and Services Administration, the Food and Drug Administration, the Administration for Community Living, the Social Security Administration, the Centers for Medicare and Medicaid Services, the Department of Defense, and the Department of Education. One-third of the members are public members, including representatives from voluntary health organizations.

**20c. How frequent and relevant are the Committee Meetings?**

As described in the MD CARE Act (P.L. 107-84), the Muscular Dystrophy Coordinating Committee meets at least twice each year. The committee met in December 2017, April 2018, and September 2018 to discuss strategies to support muscular dystrophy researchers and how to encourage new investigators to join the field, and to further discuss patient access to care and services. Meeting topics are selected with input from Committee members and are chosen for their relevance to the patient and research communities.

**20d. Why can't the advice or information this committee provides be obtained elsewhere?**

The Muscular Dystrophy Coordinating Committee is composed of both representatives of Governmental agencies and public members who have developed a plan for conducting

and supporting research and education on muscular dystrophy. The formation of the Committee was mandated in the MD-CARE Act (P.L. 107-84). The Committee will periodically review and revise the plan and will discuss ways to further coordinate activities across the NIH, with other Federal health programs and voluntary health organizations relating to the muscular dystrophies. There are no other committees with the representation and experience of the MDCC.

## **20e. Why is it necessary to close and/or partially closed committee meetings?**

N/A

## **21. Remarks**

This committee did not produce any public reports during the fiscal year. The following members were reappointed to continue to serve on the MDCC: Drs. Katz, Kiley, Koroshetz, Jillapalli, San Agustin, and Mr. Perez.

## **Designated Federal Officer**

Glen Nuckolls Program Director, Extramural Research Program

<b>Committee Members</b>	<b>Start</b>	<b>End</b>	<b>Occupation</b>	<b>Member Designation</b>
BIANCHI, DIANA	04/27/2018	12/31/2020	DIRECTOR	Regular Government Employee (RGE) Member
Cwik, Valerie	03/31/2015	12/31/2017	Medical Director and Senior Vice President for Research, MDA	Special Government Employee (SGE) Member
Denger, Brian	04/01/2015	12/31/2017	Advocate	Special Government Employee (SGE) Member
FISCHER, RYAN	08/05/2018	12/31/2020	SENIOR VICE PRESIDENT OF COMMUNITY ENGAGEMENT	Special Government Employee (SGE) Member
FREUND, EUGENE	05/23/2018	12/31/2020	MEDICAL OFFICER	Regular Government Employee (RGE) Member
Hesterlee, Sharon	06/26/2016	12/31/2018	EVP PATIENT ADVOCACY AND PUBLIC AFFAIRS	Special Government Employee (SGE) Member
Jillapalli, Devanand	01/01/2018	12/31/2020	Medical Officer	Regular Government Employee (RGE) Member
Katz, Stephen	01/01/2018	12/31/2020	Director, NIAMS	Regular Government Employee (RGE) Member
Kiley, James	01/01/2018	12/31/2020	Director	Regular Government Employee (RGE) Member
Koroshetz, Walter	01/01/2018	12/31/2019	Director, NINDS	Regular Government Employee (RGE) Member
Mittal, Plavi	04/03/2016	12/31/2018	Founder and Chief Executive Officer	Special Government Employee (SGE) Member
PAVLATH, GRACE	09/16/2018	12/31/2020	SENIOR VICE PRESIDENT/SCIENTIFIC DIRECTOR	Special Government Employee (SGE) Member
Perez, Daniel	01/01/2018	12/31/2020	President and Chief Executive Officer	Special Government Employee (SGE) Member
RICHEY, KIMBERLY	05/11/2018	12/31/2020	DEPUTY ASSISTANT SECRETARY	Regular Government Employee (RGE) Member
RILEY, CATHARINE	07/13/2018	12/31/2020	PUBLIC HEALTH ANALYST	Regular Government Employee (RGE) Member

STREET, NATALIE	05/17/2018	12/31/2020	HEALTH SCIENTIST	Regular Government Employee (RGE) Member
Salzer, Wanda	04/13/2015	12/31/2017	Director	Regular Government Employee (RGE) Member
San Agustin, M. Theresa	01/01/2018	12/31/2020	Program Manager	Regular Government Employee (RGE) Member
Spencer, Melissa	03/10/2016	12/31/2018	Deputy Associate Commissioner	Regular Government Employee (RGE) Member
Wald, Peter	04/06/2015	12/31/2017	Vice-President	Special Government Employee (SGE) Member

**Number of Committee Members Listed: 20**

### **Narrative Description**

The formation of the Muscular Dystrophy Coordinating Committee (MDCC) was mandated by the MD-CARE Act (P.L. 107-84). The purpose of the MDCC is to coordinate research activities across the National Institutes of Health (NIH) and with other Federal health programs and activities relating to the various forms of muscular dystrophy, including Duchenne, myotonic, facioscapulohumeral muscular dystrophy and other forms of muscular dystrophy. As required by law, the function of the MDCC is to develop a plan for conducting and supporting research and education on muscular dystrophy through the national research institutes and periodically review and revise the plan. The MDCC has developed a research and education plan for the muscular dystrophies for NIH. This plan lays out research goals for advancing research and treatments for the muscular dystrophies. The MDCC also has developed an Action Plan that identified key activities to be undertaken by the broader muscular dystrophy community (governmental organizations, advocacy groups, and academic and corporate investigators). This Action Plan is used as a means to identify and coordinate key activities.

### **What are the most significant program outcomes associated with this committee?**

Checked if Applies

Improvements to health or safety	<input checked="" type="checkbox"/>
Trust in government	<input checked="" type="checkbox"/>
Major policy changes	<input type="checkbox"/>
Advance in scientific research	<input checked="" type="checkbox"/>
Effective grant making	<input type="checkbox"/>
Improved service delivery	<input type="checkbox"/>
Increased customer satisfaction	<input type="checkbox"/>
Implementation of laws or regulatory requirements	<input checked="" type="checkbox"/>
Other	<input type="checkbox"/>

### **Outcome Comments**

NA

**What are the cost savings associated with this committee?**

Checked if Applies

None	<input checked="" type="checkbox"/>
Unable to Determine	<input type="checkbox"/>
Under \$100,000	<input type="checkbox"/>
\$100,000 - \$500,000	<input type="checkbox"/>
\$500,001 - \$1,000,000	<input type="checkbox"/>
\$1,000,001 - \$5,000,000	<input type="checkbox"/>
\$5,000,001 - \$10,000,000	<input type="checkbox"/>
Over \$10,000,000	<input type="checkbox"/>
Cost Savings Other	<input type="checkbox"/>

**Cost Savings Comments**

Basic and clinical research accomplishments, as supported by MDCC member organizations, often take many years to unfold into new diagnostic tests and new ways to treat and prevent diseases. Coordinated efforts by the committee allow for screening and prevention of overlapping support for research projects.

**What is the approximate Number of recommendations produced by this committee for the life of the committee?**

2

**Number of Recommendations Comments**

A meeting of the MDCC was held in March 2015 to review the revision to the Action Plan. The revised Action Plan report was approved by the committee and published on the MDCC website (mdcc.nih.gov) in November 2015. At the 2018 MDCC meetings, the Committee revisits one or more of the sub-recommendations from the 2015 Action Plan to consider progress on the recommendations and strategies to accelerate progress. This has not led to new recommendations, but enhances the committee members' understanding of current obstacles and enables them to work through their agencies and organizations to overcome the obstacles. This committee hasn't made any additional recommendations. The advice/suggestions/recommendations given at meetings are from individuals on the committee and are not voted on or developed as consensus from the group.

**What is the approximate Percentage of these recommendations that have been or will be Fully implemented by the agency?**

0%

### **% of Recommendations Fully Implemented Comments**

The Action Plan describes various research objectives that will take decades or longer to complete. But, the NIH is currently supporting and will in the future support research grants that address approximately 60% of the topics in the Action Plan (considered partially implemented, below). Due to scientific uncertainties, approximately 40% of the research objectives will be achieved (here defined as fully implemented). Approximately 40% of the Action Plan relates to topics that are beyond the mission of the NIH, but relevant to other federal agency and voluntary group members of the MDCC.

**What is the approximate Percentage of these recommendations that have been or will be Partially implemented by the agency?**

50%

### **% of Recommendations Partially Implemented Comments**

As described above, approximately 60% of the research topics and objectives described in the Action Plan are within the mission of the NIH. These research topics are or will be addressed by research grants in the future. However, not all of the scientific problems will be solved, so these are considered partially implemented.

**Does the agency provide the committee with feedback regarding actions taken to implement recommendations or advice offered?**

Yes ☒ No ☐ Not Applicable ☐

### **Agency Feedback Comments**

Memoranda to and regular communication with Committee members.

**What other actions has the agency taken as a result of the committee's advice or recommendation?**

Checked if Applies

Reorganized Priorities	<input checked="" type="checkbox"/>
Reallocated resources	<input type="checkbox"/>
Issued new regulation	<input type="checkbox"/>
Proposed legislation	<input type="checkbox"/>
Approved grants or other payments	<input type="checkbox"/>
Other	<input type="checkbox"/>

### **Action Comments**

NA

**Is the Committee engaged in the review of applications for grants?**

No

**Grant Review Comments**

NA

**How is access provided to the information for the Committee's documentation?**

Checked if Applies

Contact DFO



Online Agency Web Site



Online Committee Web Site



Online GSA FACA Web Site



Publications



Other



**Access Comments**

The MDCC website is located at: <https://mdcc.nih.gov/>. The website contains information about Committee membership, past and upcoming meetings, and contains a link to the Research and Education Plan for MD.